

112TH CONGRESS  
2D SESSION

# S. 2281

To amend the Federal Food, Drug, and Cosmetic Act to strengthen the ability of the Food and Drug Administration to seek advice from external experts regarding rare diseases, the burden of rare diseases, and the unmet medical needs of individuals with rare diseases.

---

## IN THE SENATE OF THE UNITED STATES

MARCH 29, 2012

Mr. WHITEHOUSE introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

---

## A BILL

To amend the Federal Food, Drug, and Cosmetic Act to strengthen the ability of the Food and Drug Administration to seek advice from external experts regarding rare diseases, the burden of rare diseases, and the unmet medical needs of individuals with rare diseases.

1       *Be it enacted by the Senate and House of Representa-*  
2       *tives of the United States of America in Congress assembled,*

3       **SECTION 1. SHORT TITLE.**

4       This Act may be cited as the “Expanding and Pro-  
5       moting Expertise in Rare Treatments Act of 2012” or the  
6       “ExPERT Act”.

1 **SEC. 2. FINDINGS.**

2 Congress finds as follows:

3 (1) Biomedical research is yielding discoveries  
4 that are leading to the development of new therapies  
5 that hold great promise for treating disease.

6 (2) Scientists are increasingly unlocking the po-  
7 tential for targeting treatments according to genetic  
8 defect.

9 (3) Many of the new therapies that are under  
10 development in laboratories across the Nation are  
11 targeted to rare diseases, small subsets of diseases  
12 of significant incidence, or even small subsets of rare  
13 diseases.

14 (4) Progress in the development of targeted  
15 therapies, while of great promise for those with dis-  
16 ease or disability, requires the Food and Drug Ad-  
17 ministration to develop or obtain expertise in many  
18 diseases and disease subtypes.

19 (5) In previous circumstances when the Food  
20 and Drug Administration has consulted with rare  
21 drug experts, the agency has been able to move new  
22 therapies to market with greater efficiency.

23 (6) The Food and Drug Administration benefits  
24 from this type of consultation with external experts  
25 who have a deep understanding of the diseases or  
26 disease subtypes that are targeted by new therapies.

1           (7) Access to external experts provides valuable  
2       advice about rare diseases or disease subtypes, dis-  
3       ease severity, and unmet medical needs.

4   **SEC. 3. CONSULTATION WITH EXTERNAL EXPERTS.**

5       Subchapter E of chapter V of the Federal Food,  
6   Drug, and Cosmetic Act (21 U.S.C. 360bbb et seq.) is  
7   amended by adding at the end the following:

8   **“SEC. 568. CONSULTATION WITH EXTERNAL EXPERTS ON**  
9                   **RARE DISEASES, TARGETED THERAPIES, AND**  
10                   **GENETIC TARGETING OF TREATMENTS.**

11       “(a) IN GENERAL.—

12           “(1) OPPORTUNITIES FOR CONSULTATION.—

13       The Secretary shall ensure that opportunities exist,  
14       at a time the Secretary determines appropriate, for  
15       consultation with external experts on the topics de-  
16       scribed in subsection (c), for the purpose of pro-  
17       moting the efficiency of and informing the review by  
18       the Food and Drug Administration of drugs and bio-  
19       logic products for rare diseases and drugs and bio-  
20       logic products that are genetically targeted.

21           “(2) CONSULTATION.—The Center for Drug  
22       Evaluation and Research and the Center for Bio-  
23       logics Evaluation and Research shall, when appro-  
24       priate, seek the opinion of external experts on any  
25       topic, including the topics described in subsection

1 (c), by initiating contact with such experts. External  
 2 experts may also request the opportunity to meet  
 3 with a review division regarding any topic described  
 4 in subsection (c).

5 “(b) EXTERNAL EXPERTS.—The external experts  
 6 under subsection (a) may include—

7 “(1) representatives of patient, consumer, re-  
 8 search, and health professional organizations with  
 9 expertise relevant to the review of rare disease prod-  
 10 ucts;

11 “(2) experts on rare diseases, rare subtypes of  
 12 rare and other diseases, and genetic targeting of  
 13 treatments, including experts from academia; and

14 “(3) experts in innovative clinical trial designs  
 15 for small target populations.

16 “(c) TOPICS FOR CONSULTATION.—Topics for con-  
 17 sultation may include—

18 “(1) rare diseases;

19 “(2) the severity of rare diseases;

20 “(3) the unmet medical need associated with  
 21 rare diseases;

22 “(4) the willingness and ability of individuals  
 23 with a rare disease to participate in clinical trials;

1           “(5) an assessment of the benefits and risks,  
2           including side effects, of current and investigational  
3           therapies;

4           “(6) the design of clinical trials for rare disease  
5           populations and subpopulations, including regulatory  
6           and scientific policies affecting the design of such  
7           trials; and

8           “(7) demographics and the clinical description  
9           of patient populations.

10          “(d) CLASSIFICATION AS SPECIAL GOVERNMENT EM-  
11          PLOYEES.—The external experts who are consulted under  
12          this section may be considered special government employ-  
13          ees, as defined under section 202 of title 18, United States  
14          Code.

15          “(e) PROPRIETARY INFORMATION.—Nothing in this  
16          section shall be construed to create a right for any exter-  
17          nal expert, as described in subsection (b), to obtain access  
18          to proprietary information of a sponsor without the per-  
19          mission of such sponsor.

20          “(f) NO RIGHT OR OBLIGATION.—Nothing in this  
21          section shall be construed to create a legal right for a con-  
22          sultation on any matter or require the Secretary to meet  
23          with any particular expert.”.

○